Critical Path Innovation Meetings

Guidance for Industry

DRAFT GUIDANCE

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For questions regarding this draft document contact Alicia B. Stuart 301-796-3852.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)

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Procedural

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I. INTRODUCTION

This guidance describes the purpose, scope, documentation, and administrative procedures for a Critical Path Innovation Meeting (CPIM), including how to request such a meeting. The CPIM is a means by which the Center for Drug Evaluation and Research (CDER or we) and investigators from industry, academia, patient advocacy groups, and government can communicate to improve efficiency and success in drug development. The goals of the CPIM are to discuss a methodology or technology proposed by the meeting requester and for CDER to provide general advice on how this methodology or technology might enhance drug development. CDER will identify some of the larger gaps in existing knowledge that requesters might consider addressing in the course of their work. The discussions and background information submitted through the CPIM are nonbinding on both FDA and CPIM requesters.

This guidance provides some examples of topics appropriate for a CPIM. It also describes the information that should be provided to CDER in preparation for a meeting and potential outcomes from the CPIM.

FDA's guidance documents, including this draft guidance, do not establish legally enforceable responsibilities. Instead, guidances describe the Agency's current thinking on a topic and should be viewed only as recommendations, unless specific regulatory or statutory requirements are cited. The use of the word *should* in Agency guidances means that something is suggested or recommended, but not required.

[1] This guidance has been prepared by the Office of Translational Sciences and the Office of New Drugs in the Center for Drug Evaluation and Research (CDER) at the Food and Drug Administration.

II. BACKGROUND

In 2004, FDA published the report *Innovation or Stagnation: Challenge and Opportunity on the Critical Path to New Medical Products*, which called attention to a "slowdown . . . in innovative medical therapies reaching patients." [2] The report identified several areas of product development in need of improvement, including "technical methods such as animal or computer-based predictive models, biomarkers for safety and effectiveness, and new clinical evaluation techniques," and cited a need "to create better tools for developing medical technologies [and] a knowledge base built not just on ideas from biomedical research, but on reliable insights into the pathway to patients." Through the Critical Path Initiative (CPI), FDA works to foster innovation through collaborations among government, industry, academia, patient advocacy groups, and other external stakeholders. CDER has created programs such as the Voluntary eXploratory Data Submission program (VXDS)[3] and Drug Development Tools (DDT) qualification programs[4,5] for biomarkers, clinical outcome assessments, and animal models under the Animal Rule.[6] FDA continues to work to identify opportunities to advance drug development efforts.[7]

III. SCOPE, CONTENT, AND OUTCOMES

A. Scope

The CPIM is broad in scope. It is a general discussion of challenges in drug development and innovative strategies to address them. Appropriate FDA experts from CDER offices and other centers will participate as resources and time permit.

B. Potential Topics for a CPIM

Potential topics for a CPIM include, but are not limited to, the following:

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[2] Available at www.fda.gov/ScienceResearch/SpecialTopics/CriticalPathInitiative/CriticalPathOpportunitiesReports under Challenges and Opportunities Report – March 2004.

[3] See the guidance for industry *Pharmacogenomics Data Submissions*. The guidances referenced in this document are available on the FDA Drugs guidance Web page at http://www.fda.gov/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/default.htm. We update guidances periodically. To make sure you have the most recent version of a guidance, check the FDA Drugs guidance Web page.

[4] See the Drug Development Tools (DDT) Qualification Programs Web page, http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugDevelopmentToolsQualificationProgram/.

[5] See the guidance for industry and FDA staff *Qualification Process for Drug Development Tools.*

[6] See the draft guidance for industry *Product Development Under the Animal Rule*. When final, this guidance will represent the FDA's current thinking on this topic.

[7] *Identifying CDER's Science and Research Needs Report* (2011), available at http://www.fda.gov/Drugs/ScienceResearch/ucm264327.htm.

- **Biomarkers in the early phase of development and not yet ready for the Biomarker Qualification Program (BQP)**: The CPIM can be a venue for a discussion of the potential of proposed biomarkers. The discussion can help requesters understand some of the more important questions FDA may have related to proposed biomarkers and prepare prospective submitters for the BQP.
- **Clinical outcome assessments in the early phase of development and not yet ready for the Clinical Outcome Assessment Qualification Program:** Clinical outcome assessments (COAs) include patient-reported outcomes, clinician-reported outcomes, observer-reported outcomes, and performance outcomes. The CPIM can be a venue for a discussion of the potential approaches to developing COAs to provide evidence of treatment benefit to support marketing approval and labeling claims. The discussion can help requesters understand the needs and goals for COA qualification and answer questions they may have related to the development or selection of COAs in preparation for the qualification process.
- **Natural history study designs and implementation:** The CPIM can assist in the design of natural history studies to increase the potential for the data generated by these studies to help in the design of interventional clinical trials and drug development programs.
- **Emerging technologies or new uses of existing technologies:** The CPIM may help developers understand the strengths and weaknesses of these technologies in relation to the various potential uses at different stages of drug development.
- **Innovative conceptual approaches to clinical trial design and analysis:** The CPIM can be a forum for the discussion of conceptual and general regulatory issues concerning various design and analytical approaches to clinical trials.

FDA will not give regulatory advice on specific product development programs at a CPIM. Meetings relating to a specific drug development program should be requested through the appropriate review division in accordance with the FDA guidance for industry *Formal Meetings Between the FDA and Sponsors or Applicants*. The CPIM is not intended to replace meetings that should be held through formal DDT qualification programs (see Background). Requesters interested in discussions related to the DDT qualification programs should contact the relevant DDT qualification program directly. CPIMs are not intended to discuss therapeutic product-specific data or result in binding agreements.

C. Outcomes

Through the CPIM, CDER intends to provide our perspective on the potential for use of proposed new tools and methods in drug development. Based on CDER experience, we may advise requesters of issues to consider in pursuing their work, propose joint efforts through existing consortia, or discuss the potential to form new consortia. The CPIM may also lead to recommendations for public workshops or other avenues for engaging with the wider scientific community. CDER expects that the CPIM will also provide FDA with exposure to methods and techniques that may have value in drug development.

The CPIM is not intended to be a venue for ongoing, recurrent discussions with FDA. However, we will consider requests for a subsequent meeting on a given topic on a case-by-case basis.

CDER will post the topics discussed on a quarterly basis at http://www.fda.gov/drugs/developmentapprovalprocess/druginnovation/ucm395888.htm.

IV. PROCEDURES

A. General Considerations

The CPIM is administered by the Office of Translational Sciences (OTS), which is the point of contact for all communications.

CDER will discuss with the requester, as needed, whether the issues raised in the CPIM request would be better addressed through other venues (see Scope). A request for a CPIM should not be submitted to a particular regulatory application (e.g., investigational new drug application (IND), new drug application (NDA), biologics license application (BLA)).

Requesters should clearly identify confidential or other proprietary information.

B. CPIM Requests

The CPIM request should be submitted electronically. Information about how to submit the request electronically is available at:
http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugInnovation/ucm395888.htm .

The request should include the following information:

- Name of requester
- Date of request
- Description of organization
- A document, no more than 5-6 pages in length, containing the background and purpose of the meeting, steps taken in advancing the project, and specific questions for FDA (if needed)
- Desired outcome of the meeting

Requests should provide enough information appropriate for a discussion of conceptual drug development issues and should not be focused on a particular regulatory submission. CDER plans to respond to the requester within 14 days of receipt of the meeting request and discuss with the requester the appropriateness of the CPIM.

C. CPIM Preparation Packages

If a request for a CPIM is granted, the requester should submit a final preparation package no later than 2 weeks before the meeting date.

The CPIM preparation package should be submitted to the CDER Document Room and include the following elements:

- Objective of the meeting
- Proposed agenda
- Presentation slides, if any
- Proposed attendees and respective affiliations

Send the CPIM package to the following address:

Food and Drug Administration
10903 New Hampshire Avenue
WO Building 21-4547
Silver Spring, MD 20993-0002

D. Postmeeting Summaries

We will send a meeting summary to the requester within 60 days of the meeting.